Press release
February 23, 2021

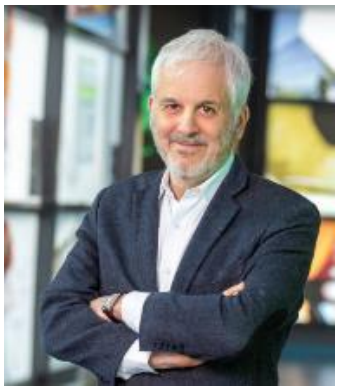
Genethon,
30 years of pioneering research and innovation in treating rare diseases

#Rarediseaseday

On February 28, International Rare Disease Day will put the spotlight on the rare diseases community throughout the world. In France, Genethon, a unique not-for-profit laboratory, innovates to develop gene therapy treatments for rare diseases.

In 30 years, Genethon has established itself as a pioneer and leader in gene therapy for rare diseases, taking down obstacles that seemed insurmountable and shaking up the therapeutic outlook for diseases long considered to be incurable. Indeed, after carrying out the first mapping of the human genome and helping identify hundreds of genes responsible for rare diseases (1991-1997), Genethon developed the highest standard of expertise in pre-clinical and clinical research and development of gene therapy treatments.

Today Genethon has 220 researchers and experts. **A unique firepower against rare diseases: 10 products from its research or to which it has contributed are in clinical trial throughout the world for rare diseases in muscle, liver, blood, the immune system and vision; 8 others should enter clinical trials in the coming 5 years.** A first gene therapy medication using technologies derived from its research received marketing authorization in the United States, Europe and Japan for type 1 spinal muscular atrophy. A second medication to which Genethon contributed in the initial R&D phases is currently being evaluated by the health authorities for Leber Hereditary Optic Neuropathy and a third is reaching the end of clinical studies and marketing approval will be requested for myotubular myopathy. Providing hope for patients and their families!



"In 1990, few of us believed that gene therapy would give rise to treatments. We were told it was too complicated, that it would never work. However, since 1997, we have built the first tools that would enable us to use genes as medications. Then, in collaboration with French and international research teams, in 2009 we showed that gene therapy could be effective in very severe rare diseases. Proofs of concept that we duplicated in rare diseases of the blood, the immune system and now the liver and muscle. Today we are faced with new challenges as we seek to overcome the scientific and technological hurdles still linked to gene therapy. We have to design more specific vectors, develop approaches for injecting these treatments and continue to innovate to treat rare diseases, as well as those said to be ultra-rare, and that should not be neglected. These are challenges for which Genethon is once again on the front lines." **Frederic Revah, CEO of Genethon**

To find out more about Genethon's pioneer work, listen to Frederic Revah's interview on Youtube

Genethon: an atypical model that fits into its ecosystem

The Genethon model is unique in the world. It emerged in 1990 out of the efforts of a patient organization - Association française contre les myopathies - thanks to funds raised during a televised charity show, a concept borrowed from the USA: the Telethon.

Genethon is a not-for-profit laboratory, whose mission is to develop gene therapy treatments for rare diseases.

Genethon works with international industrial partners to accelerate the development of its products and their availability with the aim of enabling access to treatment for all those that need it.

Genethon at a glance:

- 6,000 sq. meters of laboratory space

- 220 researchers and experts, including 5 research teams dedicated to rare diseases, and to development of technology for bioproduction

- Rare pathologies at the heart of the strategy

> Neuromuscular diseases: Duchenne muscular dystrophy, limb girdle muscular dystrophy...
> Metabolic diseases: Pompe disease, Cori disease, Crigler Najjar syndrome
> Diseases of the immune system: Wiskott Aldrich syndrome, Fanconi anemia, Chronic septic granulomatosis...

- 10 gene therapy products in clinical trial throughout the world for 10 different rare diseases

- 8 programs for entry into clinical trial in the coming 5 years

- Europe's largest DNA bank for genetic diseases: 400,000 DNA samples from 90,000 individuals for 472 different diseases

- Cutting-edge research and innovation to improve gene therapy vectors, limit immune responses and improve production

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